# Genetically Modified Hematopoietic Stem Cells for the Treatment of Danon Disease

## Grant Award Details

Genetically Modified Hematopoietic Stem Cells for the Treatment of Danon Disease

**Grant Type:** Quest - Discovery Stage Research Projects

**Grant Number:** DISC2-11131

**Investigator:**

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| **Name:** | Eric Adler |
| **Institution:** | University of California, San Diego |
| **Type:** | PI |

**Disease Focus:** Danon Disease, Heart Disease, Metabolic Disorders

**Human Stem Cell Use:** Adult Stem Cell

**Award Value:** $1,393,200

**Status:** Pre-Active

## Grant Application Details

**Application Title:** Genetically Modified Hematopoietic Stem Cells for the Treatment of Danon Disease

**Public Abstract:**

**Research Objective**

We propose to discover a novel, genetically modified hematopoietic stem cell based treatment for Danon disease, a rare lysosomal storage disease

that affects the heart.

**Impact**

As the only existing treatment for Danon disease is cardiac transplant, this therapy would significantly meet an unmet need. It also may help many other similar diseases.

**Major Proposed Activities**

- Generation of ex vivo Genetically Modified Human HSPC Product (Month 1-6)
- Functional Characterization of ex vivo Genetically Modified Human HSPC Product
- Generation of Analogous Murine Product
- In vivo Efficacy Evaluation of Analogous Murine Product in the Mouse Model of Danon Disease
- Elucidate Purported Mechanism of Action

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| **Statement of Benefit to California:** | Danon disease is a fatal disease without cure, therefore the cellular treatment we plan to develop could directly benefit the citizens of<br>California. Our findings may assist in the development of new treatments for other cardiac diseases. Thus the work also<br>has the potential to help Californians who suffer from similar cardiac conditions. This project utilizes CA scientists and laboratories. With success, it will generate additional research and employment opportunities for CA citizens. |

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